
CIRM-Funded Clinical Trial for ALS Given Go Ahead to Treat Patients

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Oakland, CA – An ingenious method that uses a patient as their own control has been given clearance to start a clinical trial to treat amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's Disease. The California Institute for Regenerative Medicine (CIRM) is funding the trial.

ALS is a devastating disease that destroys nerve cells in the brain or spinal cord. There is no treatment or cure, and the average life expectancy for someone with ALS is 3 - 4 years. Every 90 minutes someone in the US is diagnosed with ALS. Every 90 minutes someone in the US dies of ALS.

The clinical trial is led by Clive Svendsen, PhD, director of the Board of Governors Regenerative Medicine Institute at Cedars-Sinai, who has spent a dozen years developing the new approach: "Any time you're trying to treat an incurable disease, it is a long shot, but we believe the rationale behind our new approach is strong."

The new approach targets motor neurons, cells which help control movement in the body and that are killed by ALS. Svendsen and his team are using specially engineered stem cells to deliver a protein – GDNF – that is essential to the health of motor neurons. The hope is that by providing the motor neurons with GDNF they can keep them alive.

How they intend to prove that is the ingenious part. Previous studies have shown that patients with ALS experience the same rate of deterioration of movement in both legs simultaneously. Using a new device developed specifically for this trial, Svendsen intends to inject the GDNF-producing stem cells in one side of the spinal cord, into an area that controls movement in just one leg. Neither the patient nor their physicians will know which leg has received the treatment. This way the researchers will be able to study the rate of deterioration and see if it is slower in the treated leg compared to the untreated leg.

"People who have ALS face many challenges, not the least of which are the lack of treatments," says Jonathan Thomas, Ph.D., JD, Chair of the CIRM governing Board. "The disease affects around 30,000 people in the US, too small a number to make it economically viable for many companies to develop a treatment. Fortunately, the people of California created CIRM to get around problems like that. Our goal is not to make money. Our goal is to save lives. This is the kind of research that can help people with ALS and we are excited that Clive and his team are now getting the opportunity to test it in a clinical trial."

Patients eligible to be considered for the trial must be experiencing ALS-caused weakness in their legs. Implantations will take place during a five-hour surgical procedure.

Diane Winokur, the CIRM Board patient advocate for ALS, says this is truly a milestone: "In the more than 150 years since ALS was first described, there has been little progress in finding therapies. In the last few years, thanks to new technologies, increased interest, and CIRM support, we finally seem to be seeing some encouraging signs in the research. Dr. Svendsen has been at the forefront of this effort for the 20 years I have followed his work. I commend him, Cedars-Sinai, and CIRM. On behalf of those who have suffered through this cruel disease and their families and caregivers, I am filled with hope."

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to www.cirm.ca.gov

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